2019 NCCMP Annual Conference

Prescription Drug Discussion

Nick Taylor, R.Ph.
Vice President, National Pharmacy Practice Leader Consulting Services

Segal Group

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We are enthusiastic about demonstrating the areas in which we can be of assistance.

Discussion Topics

1. New Drug Overview
2. Generics and Biosimilar Drugs
3. Gene Therapy
4. Drugs in Development
5. Summary & Options
6. Q&A

Prescription Drugs

- New drug development is increasingly focused around rare disease.
- Cost of new drugs continues to increase along with concern over how to pay for these treatments.
- Ongoing legislative debate on how to reduce drug prices.
- January 2019, three dozen manufacturers announce raising prices on 250+ prescription drugs.
- 78% of all health care cost is lifestyle/behavior related.

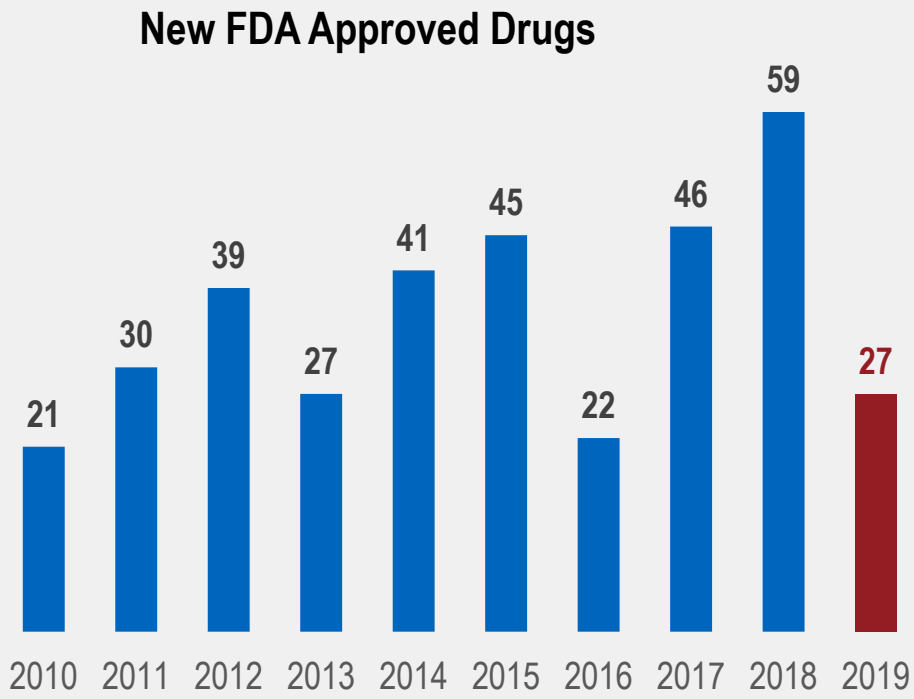

Chart is showing novel new drugs: Novel drugs are often among the more innovative products in the marketplace, and/or help advance clinical care by providing therapies never before marketed in the United States.

2019 New drugs approved through September 16.

Highlights of 2018 FDA approved New Drugs

Drugs for Rare Diseases
(22 of the 59 new approvals)

- These drugs treat rare or “orphan” diseases that affect 200,000 or fewer Americans. Patients with rare diseases often have few or no drugs available to treat their conditions.
- Examples of drugs that advance the care of patients with rare diseases approved in 2018 include:
  - **Epidiolex** (cannabidiol), for the treatment of two rare and severe forms of epilepsy: Lennox-Gastault syndrome and Dravet syndrome, in individuals two years of age and older.
  - **Palynziq** (pegvaliase-pqpz), Injection is the first FDA-approved enzyme substitution therapy designed to address the underlying cause of phenylketonuria (PKU), a rare and genetic brain-threatening condition.

Oncology
(16 of the 59 new approvals)

- Oncology drugs are used to treat a variety of cancers and represent the largest class of drugs approved.
- Examples of notable novel Oncology approvals for 2018 include:
  - **Braftovi** (encorafenib) and **Mektovi** (binimetinib) in combination to treat patients with metastatic melanoma.
  - **Vitrakvi** (larotrectinib) to treat adult and pediatric patients whose cancers have a specific genetic feature (biomarker).

New Drugs - 2019

- **Skyrizi** (risankizumab-rzaa)
  - Moderate to severe plaque psoriasis
  - Two injections every 12 weeks
  - $59,000 per patient per year

- **Rinvoq** (upadacitinib)
  - Moderately to severely active rheumatoid arthritis.
  - Taken daily as a pill.
  - $59,000 per patient per year

- **Zulresso** (brexanolone)
  - First treatment for postpartum depression in adult women.
  - 60 hour infusion given in hospital.
  - $34,000 for the drug + hospital cost

- **Vyleesi** (bremelanotide)
  - Low sex drive in women
  - Injection that needs to be administered at least 45 minutes before anticipated sexual activity.

- **Jeuveau** (prabotulinumtoxinA-Xvfs)
  - Temporary improvement in the appearance of moderate to severe glabellar lines.

- 9 of 27 approvals are for various forms of Cancer.

- One new drug each for
  - Multiple Sclerosis
  - Parkinson's Disease
  - Pneumonia
  - Irritable bowel syndrome
  - Flat worms

FDA: Record-setting year for generic drug approvals

- 1,027 new generic drugs approved
  – 843 full approvals
  – 184 “tentative” approvals
- 80 are first generic alternatives
- Priority review for up to third alternative to a brand-name drug

Generics saved consumers $1.67 trillion over the last decade

Source: U.S. Food and Drug Administration

Expected New Generics: 2019

- Expected new generics based on most current market intelligence. All subject to FDA approval and depending on drug legal challenges from brand manufacturer to block generic approval.
- It is important to note that generics may be approved but there is no requirement for a manufacturer or multiple manufacturers to produce the generic.

Anticipated Availability	Brand	Generic Name	Common Use
Q1 2019	Latuda	Lurasidone	Bipolar Depression
Q1 2019	Zyclara	Imiquimod cream	Actinic keratoses
Q1 2019	Herceptin	Trastuzumab	Breast cancer
Q1 2019	Restasis	Cyclosporine ophthalmic emulsion	Moderate to severe dry eyes
Q1 2019	Gilenya	Fingolimod	Multiple sclerosis
Q1 2019	Ranexa	Ranolazine	Angina
Q1 2019	Solodyn	Minocycline extended-release tablet	Acne
Q1 2019	AzaSite	Azithromycin 1% ophthalmic solution	Bacterial conjunctivitis
Q1 2019	Emend	Fosaprepitant injection	Nausea and vomiting
Q1 2019	Faslodex	Fulvestrant	Breast Cancer
Q2 2019	Exjade	Deferasirox tablets for oral suspension	Iron Toxicity
Q2 2019	Vesicare	Solifenacin succinate	Overactive bladder
Q2 2019	Sporanox	Itraconazole solution	Antifungal
Q2 2019	Uloric	Febuxostat	Gout
Q3 2019	Lyrica	Pregabalin	Fibromyalgia
Q3 2019	Edluar	Zolpidem sublingual tablet	Insomnia
Q3 2019	Factive	Gemifloxacin	Antibiotic
Q3 2019	Zubsolv	Buprenorphine/Naloxone sublingual tablet	Narcotic dependence
Q4 2019	Treanda	Bendamustine (powder)	Chronic lymphocytic leukemia

Biosimilar Pipeline

- Biosimilars are “generic-like” versions of biologic drugs. Biologic drugs are manufactured using a living organism, which makes it difficult to produce exact copies like other generic drugs.
- There have been several biosimilar drugs approved, however, only a few are available due to legal disputes, manufacturing issues and provider contracts.
- Overall list price for biosimilars may be up to 20% less. However contracts with medical plans and PBMs for brand products may offset this price advantage.

Innovator	Biosimilar Drug Name	Biosimilar Launch	FDA Approval Date	2018 Sales (billions)
Avastin	Zirabev™	No	Jun-2019	$6.8
	Mvasi™	Yes	Sep-2017	
Enbrel®	Erelzi®	No	Aug-2016	$5.1
	Eticovo™	No	Apr-2019	
Epogen®/Procrit®	Retacrit™	Yes	May-2018	$2.0
Herceptin®	Herzuma®	No	Dec-2018	$6.9
	Kanjinti™	Yes	Jun-2019	
	Ogivri™	No	Dec-2017	
	Ontruzant®	No	Jan-2019	
	Trazimera™	No	Mar-2019	
Humira®	Amjevita™	No	Sep-2016	$19.9
	Cyltezo®	No	Aug-2017	
	Hyrimoz™	No	Oct-2018	
Neulasta®	Fulphila™	Yes	Jun-2018	$4.4
	Udenyca®	Yes	Nov-2018	
Neupogen®	Nivestym™	Yes	Jul-2018	$0.4
	Zarxio®	Yes	Mar-2015	
Remicade®	Inflectra®	Yes	Apr-2016	$5.3
	Ixifi™	No	Dec-2017	
	Renflexis®	Yes	May-2017	
Rituxan®	Truxima®	No	Nov-2018	$1.5

Breaking Gene Therapy News

HealthAffairs

TOPICS JOURNAL BLOG

HEALTH AFFAIRS BLOG CONSIDERING HEALTH SPENDING

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Zolgensma: A Remarkable New Treatment, An ICER Analysis, And A Poorly Justified Price

Peter B. Bach

10.1377/hblog20190617.50453

JUNE 18, 2019

Defects in both sets of alleles in the coding region for t
protein lead to the rare heritable condition spinal musc
characterized by deterioration of motor neuron cells.
heterogenous, ranging from type 1 disease that cause
typically before age 2, to type 4, an adult onset condit
normal lifespan. Fewer than 1,000 infants in the U.S.

In May 2019, Novartis obtained U.S. Food and Drug A
therapy *onasemnogene abeparvovec-xioi* (Zolgensm
copies of the SMN1 gene into motor neuron cells us
remarkable single arm clinical trials, infants who ha
SMA and then received Zolgensma not only survive
but experienced improvement in neuromuscular fu
under the age of 2 with any type of SMA, whether

Hitching a ride on this broad approval was a reco
single required infusion, payable in five equal ann

THE WALL STREET JOURNAL.

YOUR HEALTH

High Hopes for a Gene Therapy Come With Fears Over Cost

Luxturna can improve sight for children and others with a rare eye condition, but costs nearly $1 million—the industry is reckoning with how to pay for such treatments as more become available

Caspian Soto plays with his parents, Krista and Zack Soto, at their home in Portland, Ore. Caspian, has a rare vision disorder and is among the first patients in the country to receive a new gene-therapy treatment. LEAH NASH FOR THE WALL STREET JOURNAL

By Sumathi Reddy

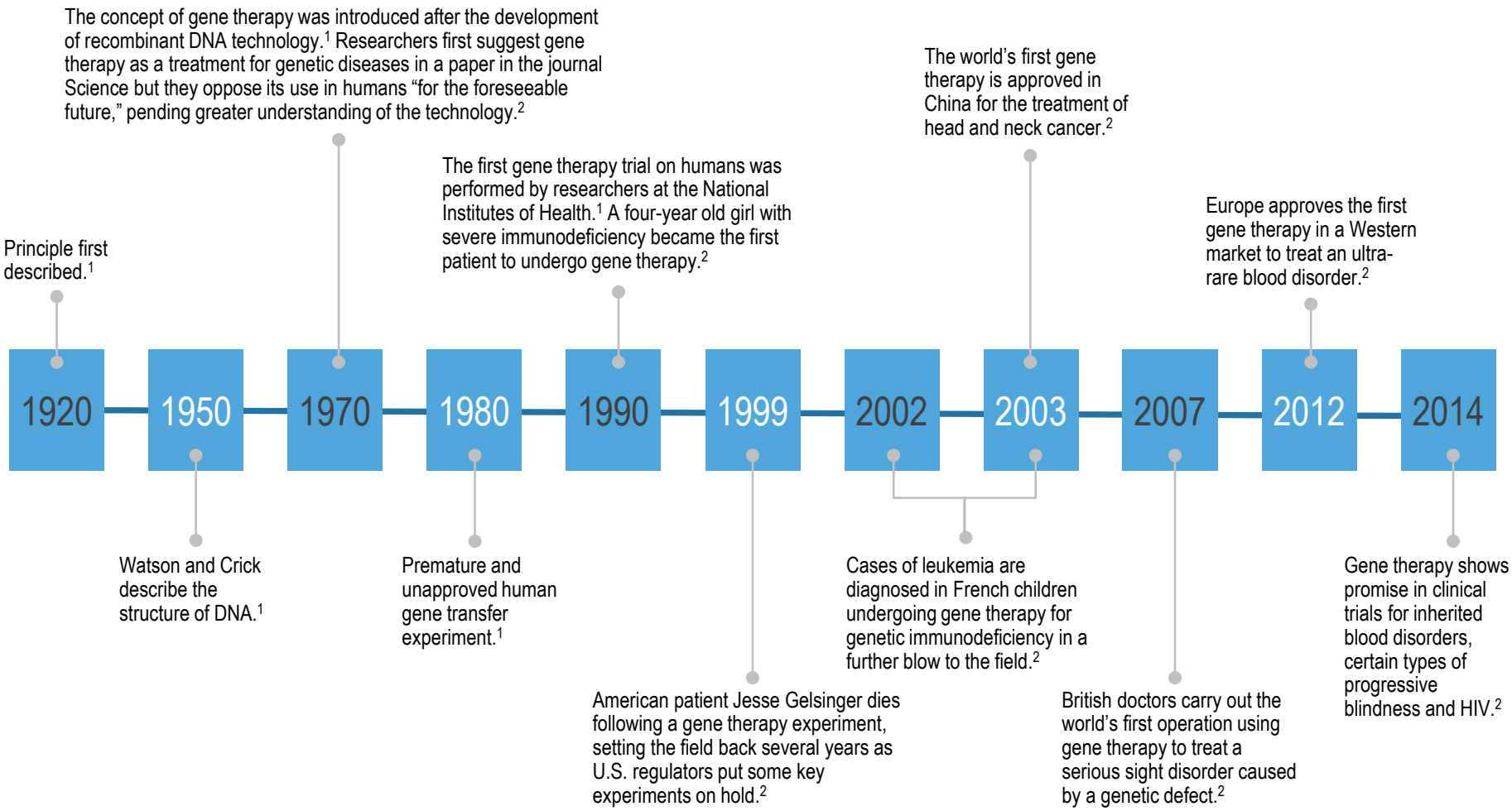
Updated Sept. 24, 2018 4:43 pm ET

SAVE SHARE TEXT 39 RESPONSES

Just 4 years old, Caspian Soto uses a cane and headlamp to help him see when he walks. He can't see in dark places like aquariums or movie theaters. He's never seen the stars.

Caspian was born with a rare, inherited eye disorder called Leber congenital amaurosis, which results in the progressive deterioration of the retina, the tissue at the back of the eye that detects light and color. He could lose all vision by the time he's a teenager.

Gene Therapy Timeline

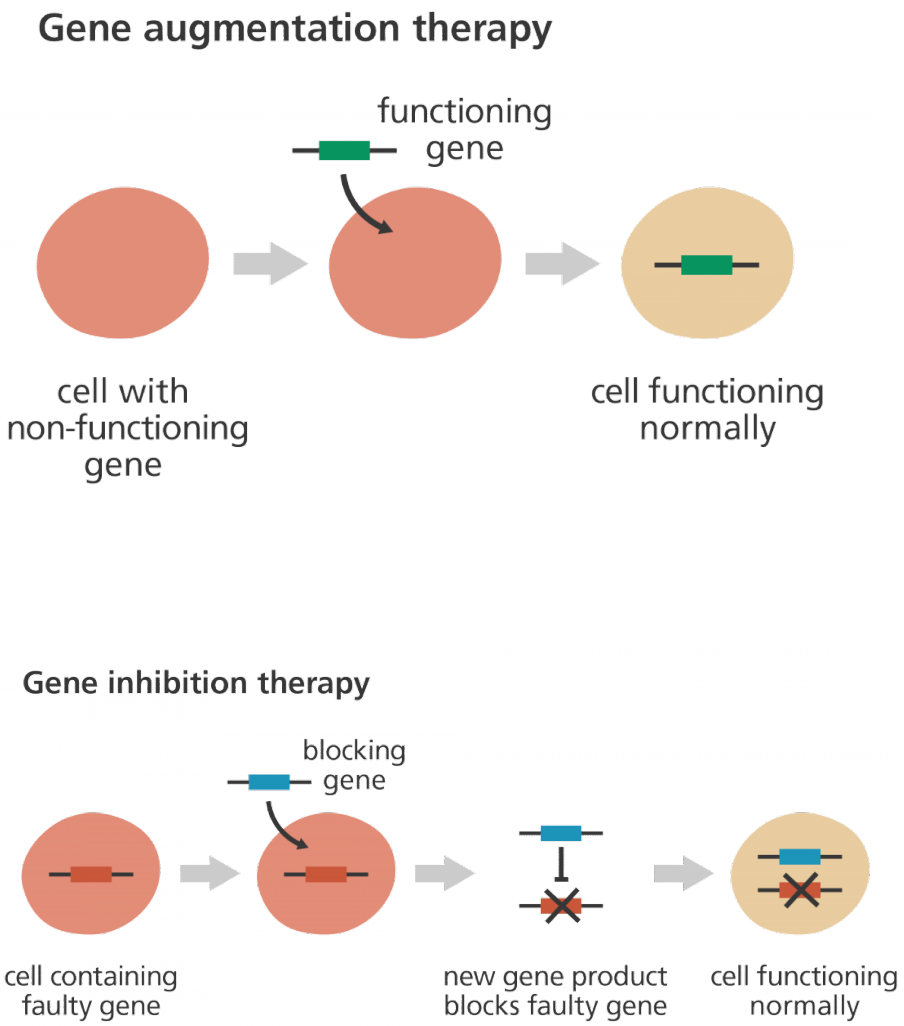


In 2017, the United States finally approved its first gene therapy, a CAR-T.

[1] www.asgct.org/about/timeline-history

[2] TIMELINE-Milestones in gene therapy (Reuters, April 27, 2015)

Gene Therapy



What to know about Gene Therapy

- Gene Therapy is a novel approach to treat, cure, or ultimately prevent disease by changing the expression of a person's genes.
- In general, gene therapy involves replacing a gene that causes a medical problem with one that does not, adding genes to help the body fight or treat disease, or turning off genes that cause medical problems.
- Cost of treatment in excess of $500,000 per patient.
- Current treatments for Leukemia, Rare Genetic Blindness and Spinal Muscular Atrophy (SMA).

Gene Therapy Pipeline

- More than 400 therapies are in development pipeline.
- Targeting primarily rare disease with limited to no treatment options.
- Technology advancements are allowing the development of highly targeted therapies.
- Growing concern over cost, payment models and commercial success.

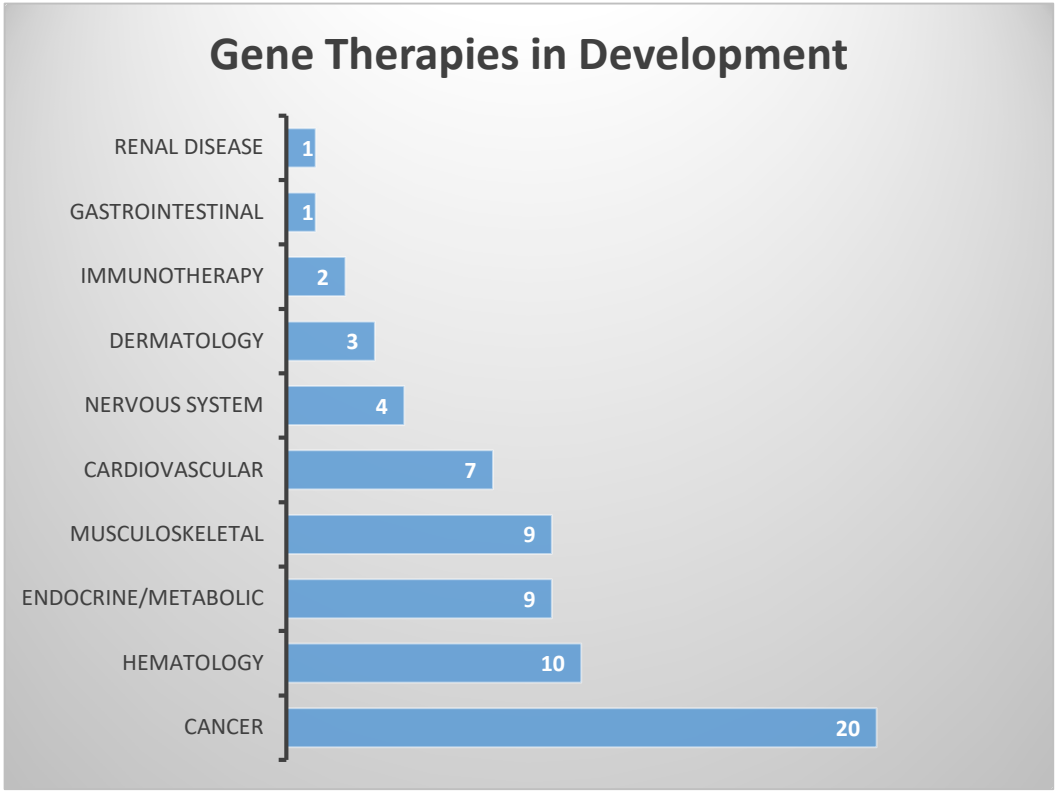


Table shows Gene Therapies in late stage development with targeted release dates between 2020 and 2022.

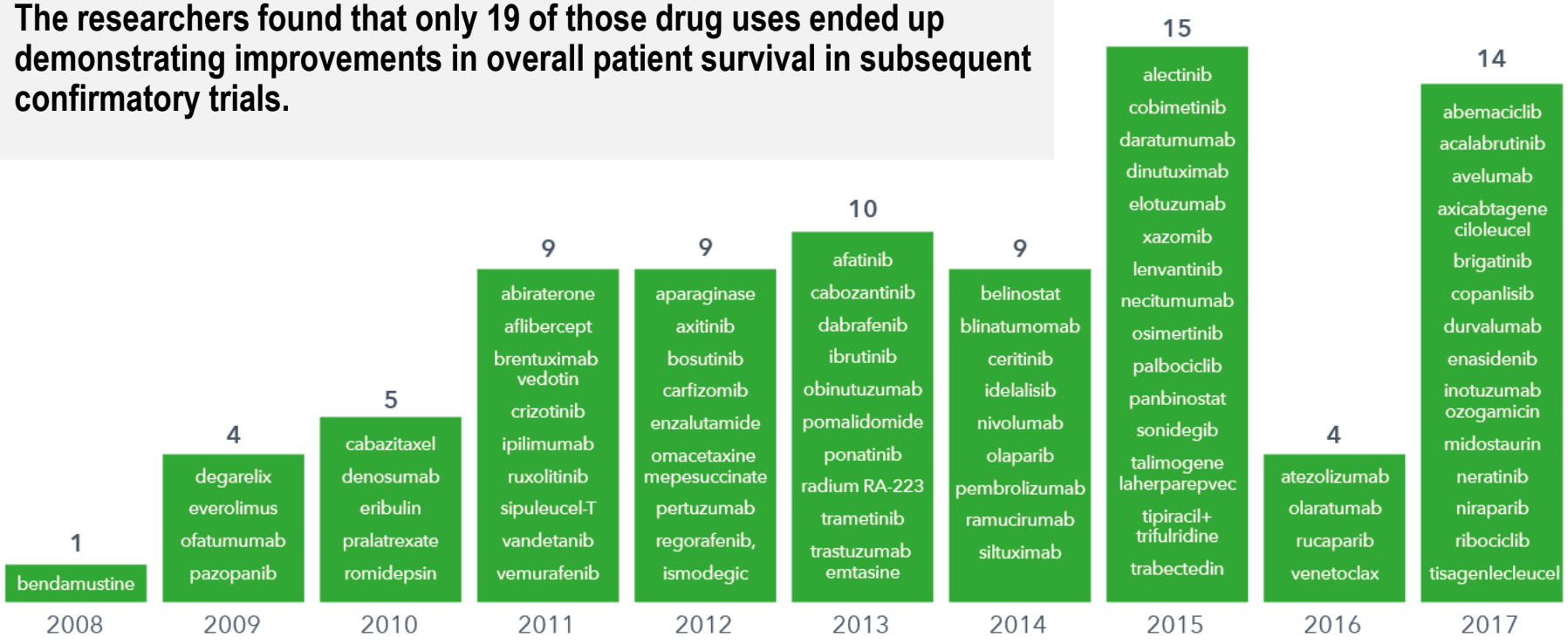
Understanding Accelerated Approval Process - FDA

- The FDA Accelerated Approval Program allows faster approval of drugs for serious conditions that fill an unmet medical need.
- The faster approval relies on use of surrogate endpoints or something that is thought to predict clinical benefit.
- Drug approvals typically require clinical trials with endpoints that demonstrate a clinical benefit.
- Surrogate endpoints typically require less time, and in the case of a cancer patient, it is much faster to measure a reduction in tumor size, for example, than overall patient survival.
- Drugs approved under the FDA Accelerated Approval Program still need to be tested in clinical trials using endpoints that demonstrate clinical benefit, and those trials are known as phase 4 confirmatory trials.
- If the drug later proves unable to demonstrate clinical benefit to patients, the FDA may withdraw approval

New Cancer Drugs

Researchers reviewed 93 cancer drug uses for which accelerated approval was granted between 1992 and 2017.

The researchers found that only 19 of those drug uses ended up demonstrating improvements in overall patient survival in subsequent confirmatory trials.

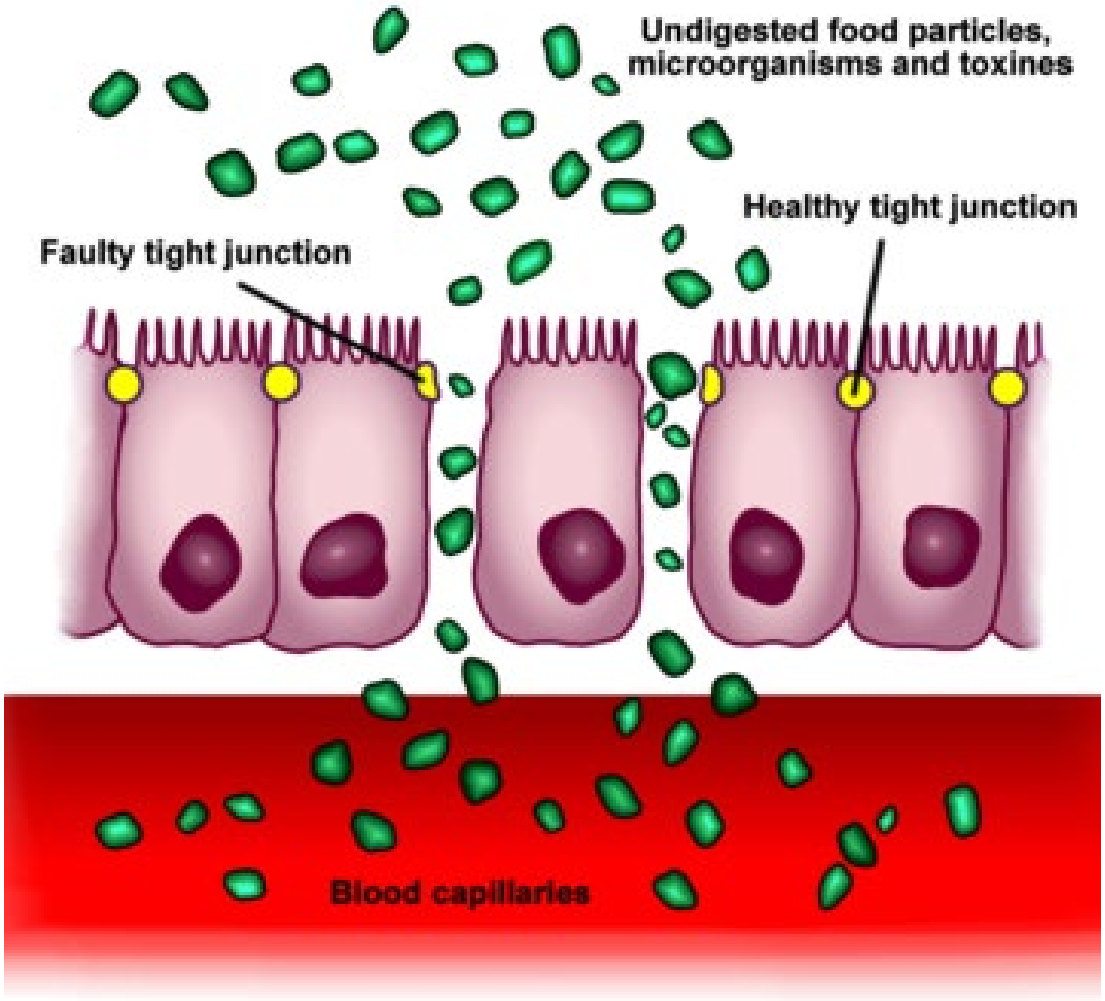


Source: IQVIA Institute, Mar 2018

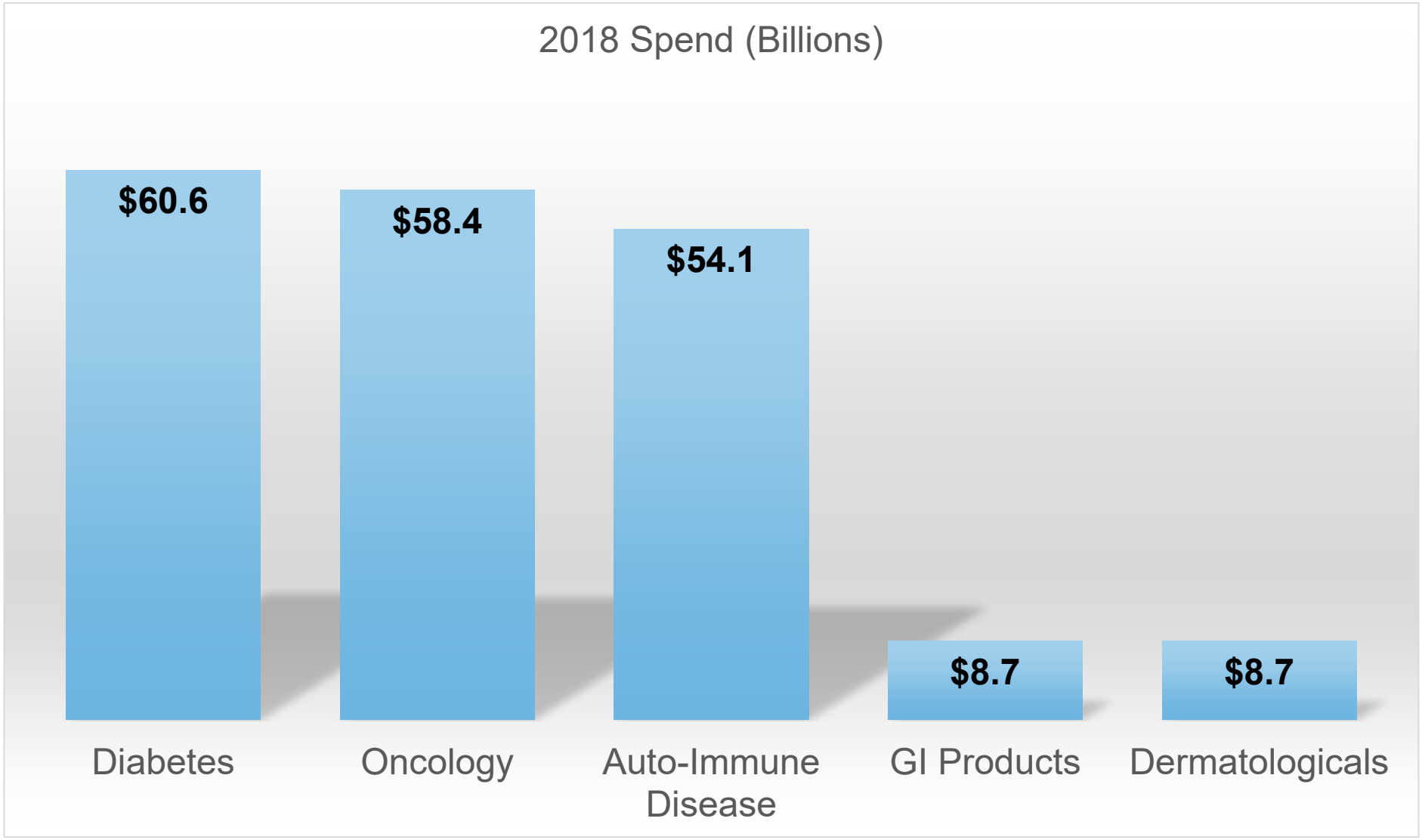
Chart notes: A New Active Substance (NAS) is a new molecular or biologic entity or combination where at least one element is new. Medicines listed by first launch year and listed alphabetically by molecular name.

Report: Medicine Use and Spending in the U.S.: A Review of 2017 and Outlook to 2022, Apr 2018

Leaky Gut Syndrome



Drug spend that can be related to Leaky Gut Syndrome



Summary and Options

- Drug innovation continues at a rapid pace primarily focused on rare diseases with limited to no treatment options.
- Drugs are coming to market faster than ever before with rising concern about overall effectiveness.
- Saving opportunities still exist with generics and biosimilar drugs.
- Plan sponsors should consider:
  - Plan design options to accommodate new drugs
  - Formularies driving to lowest net cost
  - Exclusion of high cost low value drugs
  - Clinical utilization management programs
  - Managing drug spend through both the pharmacy plan and medical plan
  - Alternative treatment programs beyond drug therapy
  - Using data analytics to identify potential risk of new therapy impact
- Be prepared to continuously evaluate new drugs and treatments!!

Thank you

Nick Taylor, R.Ph.
ntaylor@segalco.com
614-852-7170